

## SEQUENCE LISTING

&lt;110&gt; Hildinger, Markus

<120> Decreasing gene expression in a mammalian subject in vivo via  
AAV-mediated RNAi expression cassette transfer

&lt;130&gt; 2

&lt;140&gt; US 10/604,340

&lt;141&gt; 2003-07-13

&lt;160&gt; 12

&lt;170&gt; PatentIn version 3.2

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&lt;211&gt; 6437

&lt;212&gt; DNA

&lt;213&gt; Artificial

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<223> sequence for recombinant adeno-associated viral vector, including  
plasmid backbone, with AAV2 internal terminal repeats that flank  
expression cassette; referred to as AAV2/2 CMV luciferase

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&lt;221&gt; CDS

&lt;222&gt; (1228)..(2883)

&lt;223&gt; luciferase cDNA

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Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile  
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<223> sequence for recombinant adeno-associated viral vector, including  
 plasmid backbone, with AAV2 internal terminal repeats that flank  
 expression cassette; referred to as AAV2/5 CMV luciferase

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<223> luciferase cDNA

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Asp Thr Ala Ile Leu Ser Val Val Pro Phe His His Gly Phe Gly Met			
235	240	245	
ttt act aca ctc gga tat ttg ata tgt gga ttt cga gtc gtc tta atg			2022
Phe Thr Thr Leu Gly Tyr Leu Ile Cys Gly Phe Arg Val Val Leu Met			
250	255	260	265
tat aga ttt gaa gaa gag ctg ttt ctg egg agc ott cag gat tac aag			2070
Tyr Arg Phe Glu Glu Glu Leu Phe Leu Arg Ser Leu Gln Asp Tyr Lys			
270	275	280	
att caa agt gcg ctg ctg gtg cca acc cta ttc tcc ttc gtc aaa			2118
Ile Gln Ser Ala Leu Leu Val Pro Thr Leu Phe Ser Phe Phe Ala Lys			
285	290	295	

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tct ggt ggc gct ccc ctc tct aag gaa gtc ggg gaa gcg gtt gcc aag Ser Gly Gly Ala Pro Leu Ser Lys Glu Val Gly Glu Ala Val Ala Lys 315 320 325	2214
agg ttc cat ctg cca ggt atc agg caa gga tat ggg ctc act gag act Arg Phe His Leu Pro Gly Ile Arg Gln Gly Tyr Gly Leu Thr Glu Thr 330 335 340 345	2262
aca tca gct att ctg att aca ccc gag ggg gat gat aaa ccg ggc gog Thr Ser Ala Ile Leu Ile Thr Pro Glu Gly Asp Asp Lys Pro Gly Ala 350 355 360	2310
gtc ggt aaa gtt gtt cca ttt ttt gaa gcg aag gtt gtg gat ctg gat Val Gly Lys Val Val Pro Phe Phe Glu Ala Lys Val Val Asp Leu Asp 365 370 375	2358
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gcc ttg att gac aag gat gga tgg cta cat tct gga gac ata gct tac Ala Leu Ile Asp Lys Asp Gly Trp Leu His Ser Gly Asp Ile Ala Tyr 410 415 420 425	2502
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gga aag acg atg acg gaa aaa gag atc gtg gat tac gtc gcc agt caa Gly Lys Thr Met Thr Glu Lys Glu Ile Val Asp Tyr Val Ala Ser Gln 490 495 500 505	2742
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<212> PRT  
<213> Artificial

<220>  
<223> Synthetic Construct

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	15				

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	30		

Tyr Ala Leu Val Pro Gly Thr Ile Ala Phe Thr Asp Ala His Ile Glu			
35	40		45
	45		

Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala			
50	55		60
	60		

Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val					
65	70		75		80
	75				
	80				

Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu			
85	90		95
	95		

Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg			
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	110		

Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val			
115	120		125
	125		

Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro  
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Ile Ile Gln Lys Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly  
145 150 155 160

Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe  
165 170 175

Asn Glu Tyr Asp Phe Val Pro Glu Ser Phe Asp Arg Asp Lys Thr Ile  
180 185 190

Ala Leu Ile Met Asn Ser Ser Gly Ser Thr Gly Leu Pro Lys Gly Val  
195 200 205

Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp  
210 215 220

Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val  
225 230 235 240

Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu  
245 250 255

Ile Cys Gly Phe Arg Val Val Leu Met Tyr Arg Phe Glu Glu Leu  
260 265 270

Phe Leu Arg Ser Leu Gln Asp Tyr Lys Ile Gln Ser Ala Leu Leu Val  
275 280 285

Pro Thr Leu Phe Ser Phe Phe Ala Lys Ser Thr Leu Ile Asp Lys Tyr  
290 295 300

Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser  
305 310 315 320

Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile  
325 330 335

Arg Gln Gly Tyr Gly Leu Thr Glu Thr Thr Ser Ala Ile Leu Ile Thr  
340 345 350

Pro Glu Gly Asp Asp Lys Pro Gly Ala Val Gly Lys Val Val Pro Phe  
 355 360 365

Phe Glu Ala Lys Val Val Asp Leu Asp Thr Gly Lys Thr Leu Gly Val  
 370 375 380

Asn Gln Arg Gly Glu Leu Cys Val Arg Gly Pro Met Ile Met Ser Gly  
 385 390 395 400

Tyr Val Asn Asn Pro Glu Ala Thr Asn Ala Leu Ile Asp Lys Asp Gly  
 405 410 415

Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe  
 420 425 430

Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln  
 435 440 445

Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile  
 450 455 460

Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Asp Ala Gly Glu Leu  
 465 470 475 480

Pro Ala Ala Val Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys  
 485 490 495

Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Lys Leu  
 500 505 510

Arg Gly Gly Val Val Phe Val Asp Glu Val Pro Lys Gly Leu Thr Gly  
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Lys Leu Asp Ala Arg Lys Ile Arg Glu Ile Leu Ile Lys Ala Lys Lys  
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Gly Gly Lys Ile Ala Val  
 545 550

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 <211> 3618  
 <212> DNA  
 <213> Artificial  
  
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 <223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette, referred to as AAV2/5 U6 lucRI-1a

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<212> DNA  
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<220>  
<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-1b

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<210> 7
<211> 3923
<212> DNA
<213> Artificial

<220>
<223> sequence for recombinant adeno-associated viral vector, including
      plasmid backbone, with AAV2 internal terminal repeats that flank
      expression cassette; referred to as AAV2/5 U6/U6 lucRIU6-3

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<210> 8  
<211> 3589  
<212> DNA  
<213> Artificial

&lt;220&gt;

<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-4(sense)

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<211> 3589  
<212> DNA  
<213> Artificial

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<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-4(antisense)

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